UNITED STATES DISTRICT COURT
FOR THE SOUTHERN DISTRICT OF NEW YORK

Charles Seife ***Plaintiff,*** **v.** **Food and Drug Administration and Department of Health and Human Services** ***Defendants*** **and** **Sarepta Therapeutics, Inc.** ***Defendant-Intervenor*.**	**Case No. 1:17-cv-3960 (JMF)**

DECLARATION OF IAN ESTEPAN

I, Ian Estepan, pursuant to the provisions of 28 U.S.C. § 1746, declare, under penalty of perjury, as follows:

1. I am the Chief of Staff and Head of Corporate Affairs, overseeing Investor Relations, Corporate Communication, and Program Management for Defendant Intervenor Sarepta Therapeutics, Inc. ("Sarepta") in the above-captioned action. I have 16 years of experience in healthcare investing, specifically relating to the development of promising drug candidates, and over the past 5 years I have focused on speeding the clinical development of new therapies for patients with Duchenne muscular dystrophy.

2. In my current role, I am responsible for executing corporate strategic initiatives with the goal of expediting the advancement of clinical compounds through the regulatory

process, and as a result I closely track the rapidly evolving competitive landscape in Duchenne muscular dystrophy.

3. I submit this declaration in support of Sarepta's motion for summary judgment in the above-captioned action.

Background

4. Duchenne muscular dystrophy (DMD) is a serious, progressively debilitating and ultimately fatal inherited neuromuscular disease.

5. DMD affects a small population of young males, totaling approximately 9,000 to 12,000 patients in the United States.

6. DMD is caused by one or more mutations in the dystrophin gene that result in a lack of dystrophin, which is a protein that plays a vital role in the structure and function of muscle cells. The lack or near lack of dystrophin causes a progressive loss of muscle tissue and function in DMD patients.

7. Specifically, for most patients, DMD is caused by a deletion of an exon or exons to produce a "frame shift" such that the remaining exons are misaligned or "out-of-frame," interrupting proper translation of the genetic code into protein. Sequences of information in DNA are generally translated in groups of three. An out-of-frame mutation impacts the translation of every grouping of three thereafter, whereas an in-frame mutation only impacts the grouping of three containing the mutation.

8. A patient with DMD has a specific mutation profile. Research has revealed that more than half of DMD mutations involve the deletion of an exon (a sequence within a gene that will be expressed once transcribed by RNA) within the dystrophin gene, and the mutations tend to cluster between exons 45 and 55 of that gene.

9. Exon skipping is a molecular biological process by which the cellular machinery is instructed to "skip over" a certain part of a gene sequence when reading it. Exon skipping is effected by an antisense oligonucleotide that targets the mutated gene to restore the reading frame thereby facilitating translation of the genetic code into protein. In the context of muscular dystrophy, a sufficient degree of exon skipping caused by a therapeutic antisense oligonucleotide can cause the body to skip over the mutation to produce a functional, shorter form of the dystrophin gene, greatly mitigating the impact of the disease.

Sarepta's Breakthrough

10. Scientists at Sarepta created an antisense oligonucleotide platform technology that is suitable for exon skipping in a therapeutic setting. This platform technology called phosphorodiamidate morpholino oligomer ("PMO") technology is a chemically modified version of DNA to provide certain desirable properties for therapeutic exon skipping.

11. In the 2000's, Sarepta, collaborating with others, developed antisense oligonucleotides that would cause exon skipping for DMD.

12. Eteplirsen (formerly known as AVI-4658) is a PMO designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion or "exon skipping" of this exon during processing in patients with genetic mutations that are amenable to exon 51 skipping. Eteplirsen is composed of thirty subunits with each subunit having a morpholine ring, an intersubunit linkage and any one nucleobase: A, T, C or G, which taken together form a 30-nucleotide sequence that targets a specific region of the exon 51 dystrophin pre-mRNA to cause exon skipping. Being just the third antisense oligonucleotide approved by the FDA for *any* disease, eteplirsen is a complex drug with a molecular weight over 10,300 Daltons, which is over twenty

times the molecular weight of a typical small molecule drug. Eteplirsen is marketed in the United States under the brand name, EXONDYS 51®.

13. Others in the field of DMD determined that approximately 13% of DMD mutations are amenable to exon 51 skipping. DMD mutations amenable to skipping exon 51 comprise the largest subgroup of DMD patients.

Sarepta's Clinical Studies

14. Sarepta submitted the eteplirsen Investigational New Drug application ("IND") to FDA in 2007.

15. Based on promising results obtained in proof-of-concept (Phase 1) studies, Sarepta initiated a 28-week double-blind, placebo-controlled Phase 2 study at least as early as 2011. This study is referred to as Study 201.

16. Sarepta initiated a long-term Phase 2b study approximately 6 months after study 201. This study is referred to as Study 202.

17. Study 201 and Study 202 both involved the same twelve DMD patients with DMD mutations specifically amenable to exon 51 skipping.

18. The analyses from Study 201 and Study 202 included a variety of biologic and clinical outcomes. For example, changes in dystrophin were evaluated using proprietary techniques that were developed through ongoing regulatory interaction with the FDA over the course of years. Further, clinical outcomes from Study 201 and Study 202 were compared to data purchased from third parties, and used after significant quality review from Sarepta.

19. The dissemination of study results within Sarepta is carefully controlled given their potential materiality. Dissemination of study results is limited to certain members of the clinical development, regulatory, biostatistics, and data management functions, select supporting

functions, as well as certain members of the executive committee. Clinical trial sites are engaged by a CTA with terms of confidentiality. These measures are taken to preserve not only direct competitive advantages, but also to protect Sarepta's intellectual property.

20. The success of Studies 201 and 202 led to Sarepta submitting a New Drug Application ("NDA") for eteplirsen in 2015. The NDA included various analyses based on the data generated in connection with Study 201 and Study 202. These included increases in dystrophin production and the comparison of various functional outcomes compared to external controls..

21. Subsequent to the approval of EXONDYS 51® in the United States, the Company submitted a Marketing Authorization Application ("MAA") to the European Medicines Agency. The MAA is based on, among other data, comparison of various functional endpoints from Study 201, Study 202, and other studies to external controls derived from third party data obtained in part through contracts.

Competitive Harm Resulting From Disclosure of Sarepta's Clinical Study Procedures

22. Sarepta's testing protocols and procedures are proprietary and are not distributed outside of the company. Sarepta invested significant resources into developing its clinical studies and spent over three years perfecting its clinical study procedure so that Studies 201 and 202 could become a basis of approval for eteplirsen. Specific aspects of the study protocols proposed for redaction could be applied to other exon skipping drugs. Providing Sarepta's competitors with the results of Sarepta's labor would be to provide an enormous competitive advantage, both in terms of time required to advance a conceptual drug to market and the expense required to do so.

23. Sarepta recorded both these study procedures and the study outcomes in clinical study reports. These reports detail both the timing when Sarepta performed certain tests and details regarding the tests themselves. Release of this information would cause Sarepta competitive harm because Sarepta's competitors would be able to copy Sarepta's study design, or selectively modify it, without having invested the resources into producing their own study. While some details of the Sarepta test protocols have been made available, the details proposed for redaction from the requested documents have remained non-public.

24. Even basic information regarding the timing and procedures of these clinical studies constitutes highly proprietary and sensitive information because of the small DMD patient population and the dearth of therapeutics approved for DMD. Because DMD does not have previously recognized endpoints and has poorly characterized, diverse rates of disease progression, even the most elementary aspects of a clinical trial require a significant investment of resources to develop.

25. The development of Sarepta's testing procedures involved researching complicated issues such as (i) how to dose, (ii) how much to dose, (iii) how often to dose, and (iv) whether the dosing should be fixed or variable. Dosing in exon skipping therapeutics is a matter of considerable interest in the industry, and other companies, including Wave Life Sciences and Nippon Shinyaku, are currently studying dosing. Those companies are studying a variety of doses and have yet to determine a final therapeutic dose for their drug candidates. Selection of a final dose, and ultimately a competitive drug candidate, could be informed by data relating to the multiple doses and dosing administration evaluated by the Company in its studies. Were these companies to gain insights into the unpublished data regarding Sarepta's unsuccessful

and successful dosing approaches, it would allow them to bypass the years of expensive trial and error work that Sarepta undertook.

26. Another important element of Sarepta's clinical study procedure was determining the proper method to quantify dystrophin. Eteplirsen was approved by FDA through its accelerated approval pathway using dystrophin production as a surrogate clinical endpoint. Accordingly, reliably determining the amount of dystrophin protein produced in the study patients was critical to the review of the eteplirsen NDA and ultimately the basis for its approval. Dystrophin production was measured by two distinct tests. First, Sarepta developed optimized western blot techniques to determine the extent of exon skipping and amount of the skipped product. Second, Sarepta optimized immunohistochemistry ("IHC") techniques to study dystrophin localization and distribution. Being the first disease-modifying drug for DMD that was approved by FDA, Sarepta invested tremendous resources to develop procedures and protocols based on numerous FDA interactions. Many of these methods are not publicly available and have been requested by companies to aid in the rapid development and approval of competing products or other products in DMD by utilizing our proprietary techniques to establish dystrophin production. In the absence of such technology, these companies would have to develop their own validated procedures at great time and expense to demonstrate dystrophin production in a manner sufficient for regulatory approval.

27. Sarepta's research into how best to quantify dystrophin has become even more valuable because the FDA recently provided guidance that confirmed that dystrophin levels may be utilized as a surrogate endpoint to obtain accelerated approval. In order to utilize dystrophin as a surrogate endpoint, as was the case with eteplirsen, the data must be accurately and reliably produced using appropriate methods. Sarepta developed such methods over several years and

with considerable scrutiny by regulatory agencies. Making the methods, or aspects of the methods, available would provide competitor companies a basis to quickly conduct dystrophin analyses with accurate and reliable methodologies that would not otherwise be available to them and, under recent guidance, could directly result in the approval of competing drugs under expedited timelines.

28. Detailed information about each patient in the eteplirsen clinical trials and external controls - even if patient data is de-identified - would be tremendously beneficial to competitors of Sarepta not only for its own inherent value, but as an aid to designing clinical studies having a higher likelihood of succeeding thereby curtailing development ordinarily needed for FDA approval. In essence, the eteplirsen clinical trial data would facilitate a competitor to "retrospectively" design its clinical trials. Being better informed, competitors could design their clinical trials with inclusion and exclusion criteria to facilitate their success. For instance, patient age is a critical factor, as it is believed that dosing younger will give a better outcome.

Competitive Harm Resulting From Disclosure of Sarepta's Clinical Study Results

29. Publication of the results of Sarepta's testing would in effect grant Sarepta's competitors the benefit of having conducted a clinical study of eteplirsen without actually having done so. If provided access to the data tables excerpted in the clinical study report narratives or provided in full in the appendices, a competitor could simply use the results of Sarepta's clinical study to conduct a head-to-head study, without ever having administered the dosages of eteplirsen to patients as did Sarepta or run a pivotal stage clinical study. These data tables not only include individual patient level indicators but also statistical analyses of their significance.

30. The release of de-identified patient-level study results can result in competitive harm. A scientist could make productive use of the data. Change over time in clinical outcomes could be useful for purposes of powering a clinical trial, and for informing competitive decisions. DMD is a classified as an "orphan disease." It is a difficult disease to study and conduct research within due to small population size and the heterogeneous course of disease. Much has been learned about different rates of progression by specific mutations starting in 2014 with the Pane 2014 publication.. Sarepta has invested hundreds of millions in research and through this has developed proprietary knowledge that disclosure of even de-identified patient data would unfairly disclose to potential and current competitors.

31. Even when de-identified, data of this kind can contribute to development of historical external control datasets of the kind that the FDA authorized in February 2018 for use in the development of drugs to combat DMD and related diseases. Therefore release of this data will directly help our competitors build the type of control dataset that Sarepta spent years and millions of dollars producing. It is undeniable that the data resulting from Sarepta's studies, anonymized or not, represents a valuable proprietary asset, for example, the control dataset purchased and vetted by Sarepta for use in its own studies was anonymized data.

32. Third parties could also use the data to further their sales and marketing campaign to claim that its product was superior, undermine Sarepta's patent positions, or interfere with patient recruitment in subsequent studies. Providing de-identified patient level data when a competitor is not required to would put Sarepta at a competitive disadvantage. Release, with or without patient data, would allow competitors to mine the data and to characterize it in the most unfavorable light possible, whilst their own patient level data would remain safely hidden and not subject to the same potential manipulation.

33. De-identified patient-level data could also be used by a competitor as a historical control set. The FDA announced in February 2018 that while it prefers randomized placebo-controlled trials, its policy is to consider DMD trials using external controls – namely clinical studies that instead of comparing results to a placebo population, compare patient outcomes to a historical dataset of untreated similar patients that would function as an external placebo control. To use a historical control set, the drug developer must "establish that the control group was prospectively well matched to the treatment group across important baseline and prognostic variables, including age, baseline value of the primary efficacy measure and other measures of disease stage, type and intensity of supportive care, dose and duration of concomitant pharmacotherapies, and genotype, among others."[1] Sarepta utilized a historical control set for Studies 201 and 202 and incurred significant time and expense and entered into contracts in order to purchase the historical data required. Providing access to the individual patient level results of Sarepta's eteplirsen clinical studies would provide Sarepta's competitors with this information immediately and for free. Even de-identified results could be utilized as part of a historical control set. Because the general parameters of Sarepta's studies are public, such as the age range and other requirements for patient participation, the dataset could be a comparable historical comparison to a competitor's study with the same general parameters, even in the absence of individual patient demographic information. The value of this information to Sarepta's competitors is indisputable - Sarepta could sell this data for use as a control set, just as Sarepta purchased or contracted for data to utilize as its historical control set in both its NDA and MAA. Publication of this data would obviate any need for anyone to pay Sarepta for this data, leaving Sarepta without the benefit of its investment and Sarepta's competitors in the enhanced

[1] https://www.fda.gov/ucm/groups/fdagov-public/@fdagov-drugs-gen/documents/document/ucm450229.pdf

position of not only having a free historical control set but also specifically having full access to Sarepta's study results, the only similar study to result in FDA approval.

Competitive Harm Resulting From Disclosure of Sarepta's Clinical Study Endpoints

34. Sarepta performed various clinical tests on the subjects receiving eteplirsen treatment through clinical studies 201 and 202. DMD is not a well-characterized disease, and Sarepta has invested millions of dollars to further understand its characteristics. Because the disease impacts such a small population, there is little natural history data available, and the ideal outcomes for the patient population are not well-known. What would be the effect of a "successful" treatment is not commonly understood, and something Sarepta was only able to define after expending a great amount of resources.

35. Researchers, after significant study and analysis, determine which "endpoints" to use in conducting a study. Some of these, "clinical" endpoints, consider direct effects upon a patient, such as appearance (or not) of certain symptoms, performance on tests of mobility or other capability, and others. Improvement on these measures in comparison with an actual or historical control group is used as an indicator of efficacy. Other endpoints, referred to as "surrogate" endpoints, use lab measurements to track the presence or absence of a marker that is affiliated with the disease under study. Such endpoints define what is being investigated in a study, and are a critical to measure and evaluate drug efficacy.

36. In its NDA, Sarepta measured an increase in dystrophin in the body as a surrogate endpoint, and the subjects' performance on a six-minute walk test as a clinical endpoint. (CSR 201 at Bates 28-29.) In addition, Sarepta identified and investigated multiple other endpoints in both Study 201 and Study 202.

37. What endpoints a company ultimately decides to pursue in a clinical study represent not only scientific but marketing research done by company. Not all endpoints explored in a particular study are ultimately published in relation to that study. Instead, a company may choose to further research a particular exploratory endpoint in a future study, and a particular endpoint may reveal a future objective for the company's development of eteplirsen or other drugs. Unpublished exploratory endpoints would therefore be valuable to Sarepta's competitors because they provide insight into which endpoints Sarepta is pursuing, information which Sarepta's competitors could use to either mirror Sarepta's approach or to predict the areas in which Sarepta is focusing its research.

38. There is no tried and true path for developing drug candidates for DMD. To the contrary, FDA does not require companies to demonstrate DMD treatment success based on a particular defined outcome. FDA in fact encourages companies to be creative in the selection of which clinical endpoints to pursue and consider. As stated in an official FDA policy document:

> FDA has no defined set of required or recommended clinical outcome measures for studies in dystrophinopathies. Although existing outcome measures developed for clinical trials and/or clinical care in dystrophinopathies or related conditions may be appropriate, FDA will also consider proposals for the use of novel outcome measures that are capable of measuring clinically meaningful effects in patients. FDA encourages sponsors to propose and, if necessary, develop endpoints that can validly and reliably assess patients with a wide spectrum of symptoms and disease stages. Sponsors should engage FDA early during the selection and/or development of efficacy endpoints. The sponsor should include an assessment of multiple efficacy endpoints, when feasible, to characterize the breadth of effects on dystrophin-related pathologies, including skeletal, respiratory, and cardiac muscle function, even if the primary endpoint is only one of these measures.
>
> **Efficacy endpoints that can measure change of function over a wide range of types and severity of deficits may offer a number of advantages in the development of drugs for dystrophinopathies**. Such endpoints may increase the number of patients eligible for enrollment and may decrease possible loss of information from floor and ceiling effects that occur, respectively, when patients become unable to contribute data because they can no longer complete a function, or remain capable of performing a function throughout the study. For similar reasons, FDA encourages sponsors to use endpoints that can assess function across different stages of the disease (e.g., by

> combining measures of ambulation and upper body function). Endpoints should have the ability to detect improvement from baseline, as well as decline, to capture the spectrum of possible beneficial drug effects.[2]

This unique and unusual document provides a roadmap of what the FDA considers to be important in its decision of whether to approve a DMD treatment. Essential to this roadmap are clinical endpoints with a demonstrated record of improvement and how those endpoints are measured, both of which constitute highly proprietary aspects of a company's approach.

39. With knowledge of the endpoints Sarepta pursued in its clinical studies and the results Sarepta obtained, Sarepta's competitors would have an advantage that Sarepta did not when conducting its study. Whereas Sarepta individually considered, tested, and analyzed multiple potential clinical endpoints that Sarepta did not ultimately utilize to demonstrate the efficacy of eteplirsen, with knowledge of Sarepta's study into these endpoints, potential competitors would be spared the significant investment of time and resources Sarepta incurred and could simply pick up where Sarepta's research left off. This would allow competitors' products to get to market faster, and therefore to compete with Sarepta's products more imminently.

Competitive Harm Resulting From Disclosure of Nonpublic Adverse Events

40. Sarepta also recorded adverse events occurring during its clinical studies. Part of the process of performing studies like these is to determine which of these adverse events, if any, were caused by the drug. Sarepta kept careful records of the adverse events experienced in the two studies at issue, and pursued large amounts of additional data to aid in the complex and expensive task of making these distinctions. If this information were released, Sarepta's competitors would have a record of Sarepta's analysis of which adverse events occurred and did

[2] https://www.fda.gov/ucm/groups/fdagov-public/@fdagov-drugs-gen/documents/document/ucm450229.pdf

not rise to the level of drug-related adverse reactions, which they could apply to their own studies without making similar investments.

41. Because eteplirsen is the first antisense oligonucleotide approved by FDA for DMD and the first PMO ever approved, the adverse event information about the drug could be leveraged by a competitor to facilitate the approval of its own PMO for DMD irrespective of the exon. This is because a given PMO, irrespective of the exon to which it targets, generally has the same chemical backbone structure with the same DNA bases (A, T, C, G), albeit with a different order and length. As such, a competitor seeking approval for its own PMO – for exon 51 or otherwise - can leverage the eteplirsen adverse event data as being representative of the chemical class of PMO compounds. Therefore, a complete record of analysis in these studies distinguishing what events arise from that chemical action and what events do not represents an efficient shortcut to any Sarepta competitor working with exon skipping drugs.

42. Study Report 201, attachment 16.1, is a study protocol report from a Sarepta contractor that includes significant data related to the analysis of adverse events. For example, at Bates 2964, a table summarizes adverse event data from 18 different studies of chemically similar compounds. This gave the contractor a backdrop to analyze the events experienced by the patients in Study 201. Detailed charts listing and analyzing adverse events in Study 202 are found at Bates 6570-72, 6574, and 6576. Further analysis of this type including patient-by-patient descriptions of adverse events, description of analysis and testing to confirm the nature of various effects; and more. (Bates 6477; Bates 6578-6582; Bates 21650, 21652 (redacting description of procedure employed to report and identify adverse events for review).)

43. Sarepta invested heavily in research of this kind, paid the contractors it retained, and ensured that the results were not publicly released, as evidenced by the confidentiality

language found throughout the materials. Sarepta researched medical literature; retained consultants; entered into partnerships with research entities, and spent significant amounts of money and time pursuing and obtaining this information to shape its understanding of its drug. If this information is released, it will free Sarepta's competitors from a years-long process of building the necessary understanding to meaningfully study drugs of this kind.

Competition in Marketplace

44. EXONDYS 51® (eteplirsen) is currently the only exon skipping therapy approved by FDA for DMD in the U.S. The principle of exon skipping is reproducible, and other companies are using the concept to develop their own DMD treatments.

45. The public release of information regarding the application of exon skipping to DMD has led to a plethora of other pharmaceutical companies working to develop their own products. Sarepta, with eteplirsen, was the first to achieve approval by FDA to market a disease modifying drug for DMD. Since clinical development of eteplirsen was initiated, other companies have been developing antisense oligonucleotides for DMD in the US, including:

- Wave Life Sciences – Began clinical study in late 2017;
- Nippon Shinyaku – Began clinical study in the US in late 2016;
- Daiichi Sankyo – Began clinical study in the US in early 2016.

46. The following companies and institutions are also currently pursuing development of drug therapies to cause DMD patients to produce dystrophin: Daiichi Sankyo, PTC Therapeutics, Nippon Shinyaku Pharma, Wave Life Science, Solid Biosciences, Bamboo Therapeutics (acquired by Pfizer). These are Sarepta's direct competitors in this space, over which Sarepta has earned a competitive advantage by its early innovation and significant investment of company resources.

47. For example, Wave Life Sciences has begun clinical trials for WVE-210201, an investigational exon 51 skipping therapy for DMD and has announced plans to initiate clinical trials for an exon 53 skipping candidate.

48. Nippon Shinyaku is currently conducting a clinical trial of NS-065/NCNP-01, an investigational exon 53 skipping therapy for DMD.

49. Daiichi Sankyo is testing DS-5141b, an investigational exon 45 skipping therapy for DMD.

50. As stated, DMD has a very small population that is further subdivided into sub-populations, by the specific type of mutation. All companies developing DMD treatments compete for the same small patient population, and while these compounds differ significantly, testing methods are portable across exons, as is study design.

51. Companies also compete for patients to participate in clinical studies, particularly in view of the small patient numbers. DMD is rare, and the population is small. Sarepta develops antisense oligonucleotide drugs that target subgroups of the DMD population, based on their genotype. The most frequent of these genotype subgroups comprises approximately 13% of the total DMD population, and the subgroups rapidly become less frequent. Finding adequate numbers of patients to study within subgroups of an already rare population is challenging, and competitive disadvantages, or unearned advantages allowing competitors to speed up their development cycles, could hinder drug development if filling the study is deemed infeasible. Mischaracterizations of Sarepta's data by competitors could tremendously impact Sarepta's ability to enroll patients in its clinical studies.

52. Sarepta is a pioneer in the treatment of DMD using exon skipping. Sarepta's extensive clinical trial experience in exon skipping for DMD has resulted in critical advances in

our understanding of DMD, the patient population, PD and clinical endpoints, and of the mechanics of exon skipping. Further, Sarepta's understanding of DMD, the patient population, PD and clinical endpoints, and of exon skipping continues to evolve as Sarepta interrogates the vast database of clinical trial data that Sarepta has amassed as a direct result of Sarepta's extensive investments over many years in the study of DMD and continues to amass in connection with clinically advancing other exon skipping drug candidates and therapies for DMD. While other pharmaceutical companies have requested access to Sarepta's clinical trial data and testing methodologies, Sarepta has opted to maintain the confidentiality of this information.

53. The advances and continued of evolution of understanding directly informs Sarepta's strategic planning process in the design of current and future clinical trials, regulatory strategy, and competitive positioning for exon skipping and non-exon skipping therapies in Sarepta's pipeline.

54. Sarepta is currently clinically developing potential treatments for DMD amenable to exon 53 and exon 45 skipping. Golodirsen for exon 53 amenable patients, and casimersen for exon 45 amenable patients, are both being studied in a Phase 3 trial. Sarepta has preclinical drug candidates designed to skip exons 44, 52, 50, 43, 55, 8, and 35. Together, the drugs causing exon skipping of these exons could treat nearly 50% of DMD patients.

55. Sarepta is a comparably smaller than many of other companies developing DMD treatments. As such, Sarepta must build up its infrastructure globally before it can benefit from its investments in the application of exon skipping treatments to DMD. Permitting Sarepta's competitors access to detailed information regarding its clinical studies could allow Sarepta's competitors to leapfrog Sarepta in the international marketplace.

56. Eteplirsen is currently under review by the European Medicines Agency, which, if approved, would provide authorization to prescribe eteplirsen in the EU.

57. Sarepta's managed access program ("MAP") is a mechanism through which physicians can prescribe an unapproved treatment for patients with unmet medical needs, because either there is no approved treatment available in the patient's country or because treatments available in that country are not suitable to the patient. Sarepta has established a MAP in thirty countries to expand access to eteplirsen. If Sarepta's competitors with established international infrastructures are granted access to Sarepta's proprietary information, those competitors could advance their clinical development programs more quickly in these countries.

58. There are currently dozens of planned trials for other exon skipping DMD treatments. Release of the Sarepta information will accelerate the development of these trials. Not only will this represent an unearned benefit to Sarepta's competitors, but as noted it will result in a more rapid shrinking of the pool of DMD patients available for participation in Sarepta's future clinical trials than would otherwise occur, competitively harming Sarepta. (*See* ¶ 48.)

59. The competitive landscape in DMD includes 27 DMD assets in clinical development with expected US market approval between 2020 and 2027. Competition between pharmaceutical companies to work with institutions that conduct clinical studies is well established. Pharmaceutical companies invest in Medical Science Liaisons to build relationships with institutions, conduct clinical trial awareness programs and site initiation visits.

60. Clinical trial awareness programs and site initiation visits are intended to clearly communicate clinical trial related information to potential trial sites in a well-controlled manner.

Sharing of such information must be done in the proper context in order to ensure sites understand both the information, and the rationale behind the information.

I declare under penalty of perjury that the foregoing is true and correct.

Dated: April 6, 2018

By: 
Ian Estepan

Dated: April 6, 2018

Respectfully Submitted,

/s/ Daniel R. Bernstein

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